Opna Bio Announces Interim Data from Phase 1 Combination Study of OPN-2853 with Ruxolitinib in Patients with Advanced Myelofibrosis

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Preclinical Data with OPN-6602 in Multiple Myeloma Show 100% Tumor Regression in Combination Treatment Studies

San Diego, CA – December 9, 2024 - Opna Bio, a clinical-stage biopharmaceutical company focused on the discovery and development of novel oncology therapeutics, announced interim data from its lead clinical program, OPN-2853, in patients with advanced myelofibrosis showing encouraging levels of spleen reduction, with minimal toxicities and adverse events. Preclinical data from a second program, OPN-6602, showed significant tumor regression in multiple myeloma models as a single agent and in combination with other therapeutics. The presentations took place this weekend at the American Society of Hematology (ASH) annual meeting, December 7-10, 2024, in San Diego.

OPN-2853 Shows Spleen Reduction in Patients with Advanced Myelofibrosis

OPN-2853, a small molecule bromodomain and extra-terminal motif (BET) inhibitor, is being evaluated in an ongoing Phase 1 investigator-led study in patients with advanced myelofibrosis who are no longer responding to ruxolitinib alone. Myelofibrosis is a type of blood cancer that causes fibrosis in the bone marrow, anemia and enlarged spleen, amongst other symptoms. The study is testing three dose levels of OPN-2853 (20 mg, 40 mg and 80 mg), given orally once daily, in combination with ruxolitinib. As of February 2024, the cut-off date, 16 patients had been enrolled at different dose levels, across multiple sites in the United Kingdom, coordinated by the Cancer Research UK (CRUK) Clinical Trials Unit at the University of Birmingham.

In 12 evaluable patients, the median spleen size was reduced from baseline with spleens no longer palpable in 50% of evaluable patients. The combination dose has been well tolerated, and the majority of patients have completed eight cycles of combination treatment.

“We are very encouraged by these data to date, which demonstrate that daily dosing of OPN-2853 in combination with ruxolitinib was well tolerated, and showed spleen reduction in patients with myelofibrosis who have very limited options once they have progressed,” said principal investigator Adam Mead, PhD, MRCP, FRCP, professor of hematology at the University of Oxford and CRUK senior cancer research fellow. “We are enthusiastic about the OPN-2853 and ruxolitinib combination and expect to have a recommended Phase 2 dose in early 2025.”

Opna Bio is planning further clinical development with OPN-2853.

OPN-6602 Combinations Demonstrate Complete Tumor Regression in Preclinical Multiple Myeloma Models

OPN-6602, an oral, small molecule inhibitor of the E1A binding protein (EP300) and CREB-binding protein (CBP), is currently being tested in a Phase 1 trial in patients with multiple

myeloma (MM). MM is a type of blood cancer derived from malignant plasma cells in the bone marrow.

In MM mouse xenograft models, OPN-6602 showed 71% tumor suppression as a single agent, and 100% tumor regression when combined with dexamethasone, pomalidomide or mezigdomide with a sustained duration of response. RNA sequencing of treated tumors showed that OPN-6602 downregulated key MM driver and signature genes, and has the potential to overcome resistance mechanisms to standard-of-care regimens.

“We are very encouraged by the strength of the OPN-2853 clinical and OPN-6602 preclinical data, which highlights the potential of their unique pharmacokinetic profiles and validates our ‘safety by design’ approach. Both drug candidates have a high Cmax and short half-life, which allows for continuous daily dosing and effective target engagement with rapid clearance to mitigate toxicities,” said Gideon Bollag, PhD, co-founder and chief scientific officer of Opna Bio. “This confers a significant advantage to these drugs when used as single agents and a potentially synergistic effect when used in combination with other therapies.”

About Opna Bio
Opna Bio is a clinical-stage biopharmaceutical company focused on the discovery and development of novel oncology therapeutics. The company’s broad portfolio targets multiple drivers of cancer, including a novel oncology discovery program focused on the fragile-X multifunctional RNA-binding protein (FMRP) and a diversified pipeline of promising oncology assets. The Opna team has a proven track record of scientific expertise and commercial value creation, having advanced multiple FDA-approved drugs to market. Opna’s lead clinical compounds include OPN-2853, a potentially best-in-class BET bromodomain inhibitor, being evaluated in patients with myelofibrosis in combination with ruxolitinib, and OPN-6602, a dual EP300/CBP inhibitor, currently being studied in a first-in-human Phase 1 clinical trial in multiple myeloma patients. For more information, please visit opnabio.com.

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